FORMA Therapeutics today announced the initiation of a Phase 1 study of FT-1101 with the first dose administration in patients with relapsed/refractory acute myeloid leukemia (AML) or high-risk myelodysplastic syndrome. FT-1101 is an oral, structurally distinct and potent pan-inhibitor of the BET (Bromodomain and Extra-T erminal) epigenetic protein family.

“The BET protein family represents an attractive group of therapeutic targets for a variety of liquid and solid tumors, as inhibition of these epigenetic binding proteins allows for selective effects on gene expression. FT-1101 is a novel, oral small molecule targeted against all four BET family members (BRD2, BRD3, BRD4, BRDT),” said John Hohneker, M.D., EVP and Head of Research and Development, FORMA Therapeutics. “The launch of this study is an important step for our team, and we are eager to begin the selection of preferred dosing schedules and potential patient populations in order to optimize activity and tolerability of this novel medicine.”

Small molecule inhibition of BET results in down-regulation of the critical oncogene MYC, a master regulator of diverse cell functions critical for cell growth and survival in many cancers. At tolerated doses in human tumor xenograft mouse models, FT-1101 has demonstrated significant anti-tumor activity including tumor regressions.

FT-1101 is part of FORMAs second global strategic collaboration with Celgene Corporation announced in April 2014. Celgene has obtained an exclusive EU license for FT-1101 in exchange for an undisclosed payment to FORMA. Under the terms of the collaboration agreement, FORMA will advance the FT-1101 program through Phase 1, and Celgene will be responsible to fund and execute further global clinical development.

“I am impressed with the inhibition profile and differentiated preclinical activity of FT-1101, and we aim to move quickly to define the safety, tolerability and initial activity of this novel BET inhibitor in acute leukemias and myelodysplastic syndrome,” said Guillermo Garcia-Manero, M.D., lead clinical investigator for FT-1101 at The University of Texas MD Anderson Cancer Center, Houston, TX, and Professor, Department of Leukemia, Division of Cancer Medicine.

About the Study

The Phase 1 multicenter, open-label, dose escalation clinical trial is designed to assess the safety and tolerability of FT-1101 capsules as a single agent. FT-1101 will be administered orally on a once weekly dosing schedule in a 28-day cycle. The study will enroll patients with relapsed refractory AML and high risk myelodysplastic syndrome. Key objectives in the study include determination of a maximum tolerated dose, pharmacokinetics, pharmacodynamics and preliminary anti-tumor activity of FT-1101. Please refer to www.clinicaltrials.gov for additional clinical trial details.

About BET

The Bromodomain and Extra-T erminal (BET) family of bromodomain-containing proteins (BRD2, BRD3, BRD4, and BRDT) regulate chromatin structure and gene expression through their ability to bind to acetylated lysine residues on histone tails and act as epigenetic readers. In particular, BRD4 has been shown to positively regulate the expression of MYC and other critical cancer-associated genes through the localization of BRD4 to super-enhancer regulatory elements. Preclinical studies conducted using BET inhibitors and emerging data from ongoing clinical trials in leukemia and lymphoma patients, have provided initial support for the therapeutic potential of BET inhibitors in hematologic malignancies.

About FORMA

FORMA Therapeutics’ scientists are passionate about discovering and developing medicines that will make a difference in oncology, inflammation & immunity, and other serious diseases. The Company’s fully integrated R&D team drives discovery and early clinical development of therapeutics for qualified targets in the areas of epigenetics, protein homeostasis and metabolism. Leveraging a world class network of academic investigators, clinical experts and partners, FORMA combines deep biology insight, chemistry expertise and early clinical development capabilities, to create drug candidates that will ultimately provide profound patient benefit.

FORMA is headquartered in Watertown, MA near the epicenter of the Cambridge Life Sciences cluster, with additional chemistry operations in Branford, CT. For more information, please visit www.formatherapeutics.com, LinkedIn, or join our conversation on Twitter @FORMAInc.

About Celgene

Celgene Corporation, headquartered in Summit, New Jersey, is an integrated global pharmaceutical company engaged primarily in the discovery, development and commercialization of innovative therapies for the treatment of cancer and inflammatory diseases through gene and protein regulation.

For more information, please visit www.celgene.com. Follow Celgene on Social Media: @Celgene, Pinterest, LinkedIn and YouTube.

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